Health Outcome Assessment in Patients with Pulmonary Arterial Hypertension Treated with Riociguat: 1-Year Results from the PATENT-2 Long-Term Extension Study

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BACKGROUND: Pulmonary arterial hypertension (PAH) is a chronic and progressive disease that impairs physical function, often resulting in diminished health-related quality of life. The European quality of life 5 dimensions (EQ5D) questionnaire is a widely used standardized instrument that measures patient-reported health outcome in five dimensions (mobility, self-care, usual activities, pain/discomfort, and anxiety/depression) and yields a single summary health status or utility score ranging from 0 to 1. In a recent Phase III study (PATENT-1) riociguat demonstrated robust efficacy in PAH. In the open-label extension phase (PATENT-2) long-term treatment with riociguat in patients with PAH was investigated. Here we report the 1-year health outcome data from PATENT-2.

METHODS: In PATENT-1, patients with symptomatic PAH were randomized 2:4:1 to the three treatment arms: placebo, riociguat 2.5 mg–maximum group, and riociguat 1.5 mg–maximum group. Eligible patients, who completed PATENT-1 without any ongoing riociguat-related SAEs, entered the open-label PATENT-2 extension phase wherein both placebo and riociguat 1.5 mg maximum groups were switched to a maximum of 2.5 mg over an 8-week blinded phase. Health-related quality of life was assessed using the EQ5D score in both PATENT-1 (baseline and Week 12), and PATENT-2 (Week 12, and months 6, 9, 12, and 24). Descriptive statistics and 95% confidence intervals (CI) for the entire study sample were used to summarize the health outcomes for each arm across both PATENT-1 and PATENT-2. This presentation reports EQ5D data in the first year of PATENT-2.

RESULTS: A total of 443 patients underwent randomization and received at least one dose of riociguat or placebo. Out of these, 396 (89%) entered PATENT-2. At baseline, patients entering PATENT-2 were on average 50 years of age, 80% were female, 50% were treatment-naïve, and 50% were receiving treatment with ERAs or prostanoids in combination with riociguat, mean six-minute walking distance was 367 meters and 43% and 54% in functional class II and III, respectively. At the time of these data analyses, EQ5D was available for 392 (99%) and 330 (83%) patients at baseline and month 12, respectively. The EQ5D utility score for the entire study sample was 0.68 (95% CI: 0.66–0.71) at baseline and 0.75 (95% CI: 0.73–0.78) at Month 12. In the treatment-naïve subgroup, the overall baseline and Month 12 health utilities were 0.69 (95% CI: 0.65–0.72) and 0.75 (95% CI: 0.71–0.78), respectively. When riociguat was used in combination with ERAs or prostanoids, health utility increased from 0.68 (95% CI: 0.65–0.71) at baseline to 0.76 (95% CI: 0.73–0.79) at Month 12.

CONCLUSIONS: Patients with PAH treated with riociguat showed an improvement in their health status over a 12-month period.